

# SOMETHING BETTER THAN HOPE

## Strategic Concepts for Beyond 2020



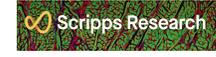
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June 26, 2020

# Leveraging Institutional Knowledge to Inform Strategic Concepts:

- ❖ CIRM's 14 year experience with >3000 grants reviewed and >1000 awards managed
- ❖ CIRM has served on academic and industry leadership forums and published on policy, industry and science
- ❖ Continual Engagement with Community, Academic, Business, Industry, Policy Networks, CIRM reviewers, Patients, Expert Advisors, ICOC



The National Academies of SCIENCES • ENGINEERING • MEDICINE



Multi-Stakeholder Engagement for Policy & Research Standards



Conferences

2018 iPSC Keystone Symposium



Audacious Goals Initiative (AGI) for Regenerative Medicine in Vision



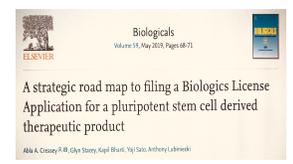
2019 GWG Program Meeting

Jan 2020 Patient Navigation Workshop



Forum Translating Science into the Clinic: The Role of Funding Agencies

Gilberto R. Sambrano · J. R. Miller · Maria T. Millan · J. R. Miller



CIRM Workshops

Annual Alpha Clinics Symposium

2018 Manufacturing Workshop



BRAINSTORMING NEURODEGENERATION

CIRM Perspectives & Whitepapers



PERSPECTIVES | Open Access

Unleashing the cure: Overcoming persistent obstacles in the translation and expanded use of hematopoietic stem cell-based therapies

Sohel Talib · Kelly A. Sheppard

STANDARDS, PROTOCOLS, POLICIES, AND REGULATIONS FOR CELL-BASED THERAPIES

Regulated, reliable, and reputable: Protect patients with uniform standards for stem cell treatments

Geoffrey P. Lomax · Art Torres · Maria T. Millan



2019 Neurodegeneration Workshop

Leveraging Genomics, Stem Cells, Gene Therapy and Novel Clinical Trials for Field-wide Advancement



Community Outreach



## Proposed Framework for Strategic Planning Process

### Feb 2020 ICOC: Introduction of Strategic Themes

- Advance World Class Science
- Build Pathways to Commercialization
- Increase Patient Access to Innovative Treatments
- Maximize Impact through Operational Excellence

### June 2020 ICOC: Board Discussion and Input re. Strategic Themes & Concepts

H2 2020: “Deep Dive” into Strategic Concepts with Board, patient advocates, external advisors and key opinion leaders

Q4 2020: Draft Outline for Strategic Plan for further development pending outcome of

**- November 2020 Ballot Initiative -**

Q1 2021 ICOC: Draft Strategic Plan

H1 2021 ICOC: Adoption of Strategic Plan for CIRM Beyond 2020



# Progress on 2016-2020 Strategic Plan



## DISCOVER

46\* of target 50  
NEW CANDIDATES

*\*48 if COVID awards approved for funding*



## ADVANCE

72 progressing  
THROUGH  
DEVELOPMENT  
100% increase

## REFINE REGULATORY

6 RMATs  
Under  
21<sup>st</sup> Century Cures



## SHORTEN TIME TO CLINICAL TESTING

72% CLIN1  
achieved IND in 2  
years

55% achieved IND  
in <18 months



## EXPAND

46 of target 50  
NEW TRIALS  
(63 Total Trials  
>2000 Enrolled)

*\*47 if COVID award approved*



## INCREASE INDUSTRY PULL

59% partnered  
63 events  
\$8.74 B Industry investment  
(of total \$10.3 B leverage)

# CIRM Beyond 2020



# Where are we today?

Mission: To accelerate stem cell treatments to patients with unmet medical needs

## CIRM's Value Proposition:

Proven acceleration-based Funding Partnership Model

Robust portfolio of 1000 programs

63 Clinical Trials

Diverse technology platforms

"De-risk" promising science-- >\$8B in Industry Partnerships to date

Enable and enact the evolving regulatory paradigm

Specialized Infrastructure- Alpha Clinics, Genomics Data Hub, Translational Hub

Education & Training Programs have seeded the new field

Patient & community advocate leadership shape the agency



Where do we want to be?

- Accelerate the development of near term and future innovative regenerative medicine approaches to deliver effective cures and treatments
- Expand therapeutic delivery and equitable access to patients with unmet medical needs
- Partner with key stakeholders and structure internal operations to accomplish the above



# Leverage CIRM's Value Proposition:

CIRM's acceleration-based funding model to

- Increase impact of each project that is funded
- Provide built-in knowledge capture
- Incorporate new paradigms including vital research opportunities
- Design programs so that success in any given project progresses the overall CIRM pipeline & that line of research

How do we get there?

Formalize strategic alliances and partnerships with external stakeholders to enhance our impact

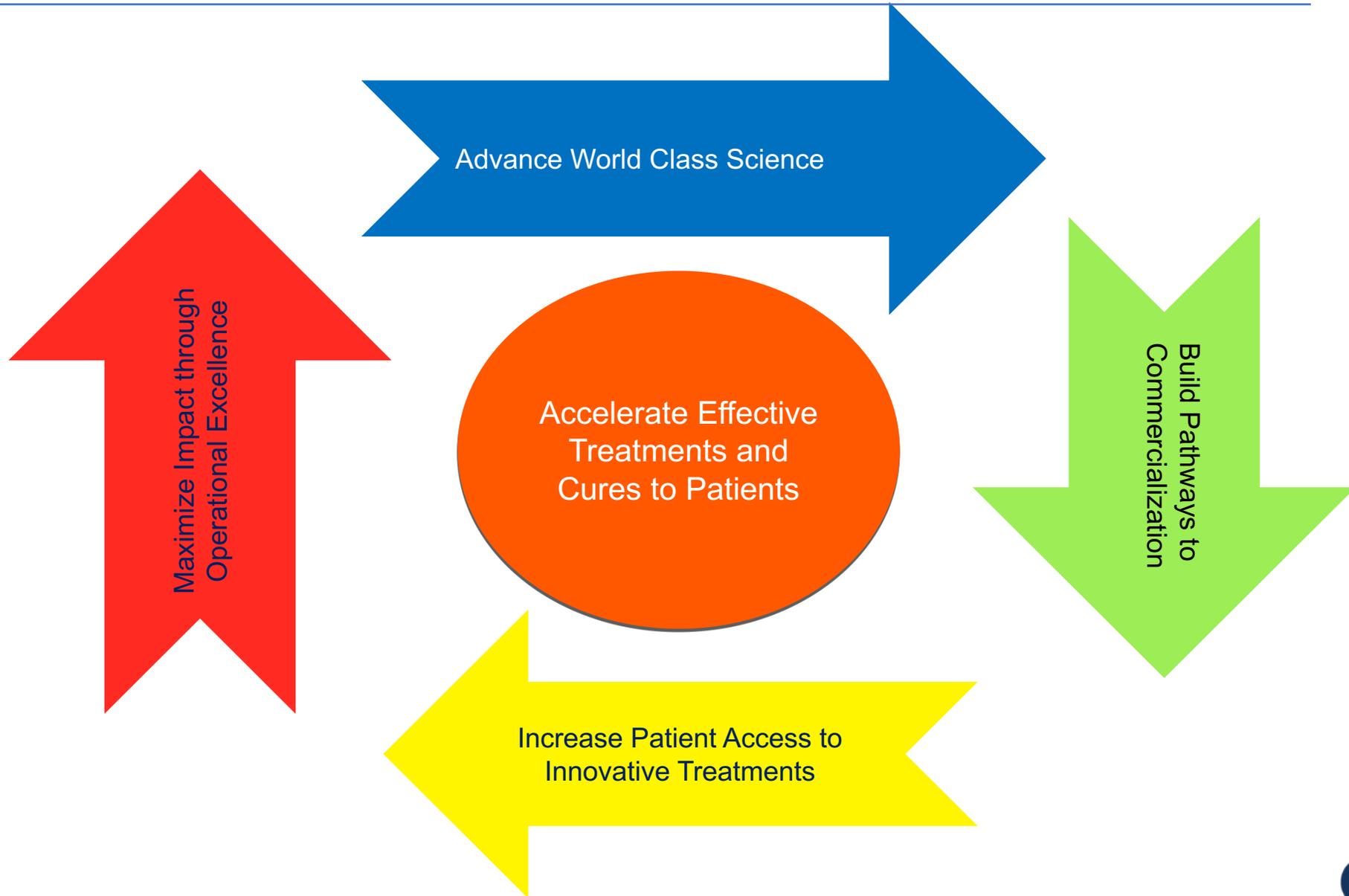
Leverage CIRM's role in "de-risking" projects and attracting Industry Investment to create commercialization pathways and post approval success of CIRM portfolio

Partner with and empower community stakeholders to optimize access to novel investigative and approved therapies



# Strategic Themes: How do we get there?

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# Theme 1: Advance World Class Science

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- Build on and Utilize the 5 Pillars: DISC,TRAN,CLIN,EDU,INFR
- Data Sharing and Data Access built into funding model
- Lateral integration of pillars and vertical integration of funding opportunities to enable consortium model and "Moon Shot"
- A proactive "Quality by design" approach for scientific programs (from DISC2-TRAN-CLIN) so they are "development ready" for regulatory pathway to clinics and "tech transfer" ready for mfr. scale up & commercialization

# Theme 1: Advance World Class Science

<p>Shared Resources &amp; Novel Technologies</p>	<p>Accelerate Science Continually test &amp; validated</p> <p>Built-in collaborations and access to CIRM programs</p>	<p>Technology exchange networks through Core labs and “built in” access for funded programs creating “win win” for technology developers and technology users--- both advancing research</p> <p>e.g. cell models, specialized assays/readouts</p> <p>Knowledge Networks and Data Sharing</p>
<p>Education &amp; Training</p>	<p>Workforce Development</p>	<p>Education Programs feed into workforce development pathways, certification and laterally integrated with other programs (e.g. core labs, manufacturing programs, clinical networks, research awards)</p> <p>Training and Certification to seed a novel field: Manufacturing, Regenerative Medicine Fellowships for Physician &amp; Scientists, Nursing, Patient Navigators, Community Educators</p>
<p>Clinical Paradigm</p>	<p>New regulatory paradigms &amp; next generation trial design</p>	<p>New Clinical Programs: Platform Trials, Umbrella Trials, Phase IV Trials, Healthcare Economics Research</p>
<p>Strategic Partnerships</p>	<p>Synergy to yield acceleration and measurable outcomes</p>	<p>Co-funding &amp; complementarity in resources and stakeholders e.g. funding agencies, foundations, public-private</p>

# Theme 2: Build Pathways to Commercialization

## Industry Partnerships to Date

2015

2016

2017

2018

2019

2020



*\*includes licenses, options, partnerships, private/public market financings*

# Theme 2: Build Pathways to Commercialization

Create Best in Class Investment & Commercialization Partnerships	Facilitate “quality by design” and accelerate project progression	Leverage advisory panels & internal expertise
		Leverage Industry Alliance Program for early support & shared de-risking & downstream commercialization partnership
Attract & Facilitate Economic Development in CA for Regenerative Medicine	Create capacity to address key gating items to commercialization	Public-Private manufacturing partnerships
Partner with funded Institutions to enhance Value Creation in CIRM-funded Projects	Promote IP Development & Business Development	Partner with institutional OTLs to optimize value and attract best in class commercialization partners for CIRM programs

# Theme 3: Increase Patient Access

Patient Navigation*	Data Registry and Educational Resources
	Training and Certification program for Patient Navigators and Counselors
	Leverage the Power of CIRM Clinical Networks to empower Community Centers to enable adoption and safe application of regulated and validated regenerative medicine treatments
Community Partnership*	Expand outreach to complete a community “feedback loop” to shape CIRM Programs
	Create novel funding mechanism for public health research- e.g. social determinants of health, historical & current barriers to healthcare delivery, access to regenerative medicine treatments
Treatment Access and Coverage*	Build internal expertise in healthcare economics, market access, insurance coverage Anticipate new RFAs and RFPs to address access Lateral integration with CIRM clinical programs Internal Team will work with the newly formed Accessibility and Affordability Working Group and the ICOC Legislative subcommittee

\* All programs designed to address the needs of the underserved, minority and vulnerable populations

# Theme 4: Maximize Impact Through Operational Excellence

Recurring RFAs	Re-open standing DISC/TRAN/CLIN RFA's while new concepts and refinements are being developed
Implement Strategic Themes	<p>Through application, review and contracting levers, and IT &amp; Data architecture, CIRM Operations will serve as cable to integrate and standardize approach for:</p> <ul style="list-style-type: none"><li>Data Sharing</li><li>Consortium Approach and future “moonshot” programs</li><li>Strategic Partnerships (precedent- NHLBI co-funding partnership)</li><li>Optimizing use of core labs and infrastructure where appropriate</li></ul> <p>Integrate and standardize new elements into application, review &amp; tracking:</p> <ul style="list-style-type: none"><li>Novel Clinical Paradigms and Study Design</li><li>Healthcare Economics</li><li>Business and Commercialization elements</li><li>Inclusion of under-represented, underserved, minority and vulnerable communities</li></ul>
Additional Funding & Review Formats	Renewal applications, Supplemental funding, Planning grants, Targeted RFAs, Infrastructure and Special RFA GWGs

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